

CLAIMS:

1. A recombinant nucleic acid molecule comprising a first sequence encoding a transgene under the control of regulatory sequences that direct expression of said transgene in a hematopoietic stem cell, a progenitor cell or cell differentiated therefrom.
2. The molecule according to claim 1, wherein said differentiated cell is a secretory cell.
3. The molecule according to claim 1, wherein said progenitor cell is selected from the group consisting of common lymphoid progenitor, common myeloid progenitor, megakaryotic/erythrocyte progenitor and granulocytes/macrophage progenitor.
4. The molecule according to claim 3, wherein said cells differentiated from megakaryotic/erythrocyte progenitor cells are selected from the group consisting of platelets, megakaryocytes and erythrocytes.
5. The molecule according to claim 3, wherein said cells differentiated from granulocyte/macrophage progenitor cells are selected from the group consisting of neutrophils, eosinophils, monocytes, basophils and immature dendritic cells.
6. The molecule according to claim 3, wherein said cells differentiated from said lymphoid progenitors are natural killer cells.
7. The molecule according to claim 5, wherein said cells differentiated from said monocytes are selected from the group consisting of mast cells, macrophages and dendritic cells.

8. The molecule according to claim 1, wherein said nucleic acid molecule is a viral or non-viral vector.

9. The molecule according to claim 1, wherein said regulatory sequence is a platelet-specific promoter.

10. The molecule according to claim 9, wherein said promoter is selected from the group consisting of the Platelet factor 4 promoter, the glycoprotein IIb promoter, the glycoprotein IIIa promoter, and the glycoprotein VI promoter.

11. The molecule according to claim 1, wherein said regulatory sequence is a neutrophil-specific promoter.

12. The molecule according to claim 11, wherein said promoter is selected from the group consisting of DEFA1 human neutrophil alpha defensin promoter, the DEFA2 human neutrophil alpha defensin promoter, DEFA3 human neutrophil alpha defensin promoter, and the DEFA4 human neutrophil alpha defensin promoter.

13. The molecule according to claim 1, wherein said regulatory sequence is a natural killer cell-specific promoter.

14. The molecule according to claim 13, wherein said promoter is the human perforin gene promoter.

15. The molecule according to claim 1, wherein said regulatory sequence is an eosinophil-specific promoter.

16. The molecule according to claim 15, wherein said promoter is selected from the group consisting of the human eotaxin gene promoter and the eosinophil peroxidase gene promoter.

17. The molecule according to claim 1, wherein said regulatory sequence is an erythrocyte-specific promoter.

18. The molecule according to claim 17 wherein said promoter is the
5 human RhD gene promoter.

19. A hematopoietic stem cell transformed, transduced, infected or transfected with a nucleic acid molecule of claim 1.

20. A host cell differentiated from a hematopoietic stem cell transformed, transduced, infected or transfected with a nucleic acid molecule of claim 1.

21. The host cell according to claim 20, selected from the group consisting of a common lymphoid progenitor cell, a common myeloid progenitor cell, a
15 megakaryotic/erythrocyte progenitor cell, a granulocyte/macrophage progenitor cell, platelets, megakaryocytes, neutrophils, eosinophils, monocytes, basophils, dendritic cells, mast cells, macrophages, dendritic cells, erythrocytes, and natural killer cells.

22. A platelet transformed, transduced, infected or transfected with a
20 nucleic acid molecule comprising a first sequence encoding a transgene under the control of regulatory sequences that direct expression of said transgene in said platelet.

23. A method for generating a modified hematopoietic stem cell, modified progenitor cell or a modified cell differentiated from said stem cell or progenitor cell
25 comprising the step of transferring a nucleic acid molecule of claim 1 into said cell via transformation, transduction, infection or transfection.

24. The method according to claim 23, further comprising the step of harvesting said stem cells or progenitor cells of the hematopoietic lineage from bone
30 marrow of a mammal prior to said transferring step, wherein said transferring step occurs *in vitro* or *ex vivo*.

25. The method according to claim 23, further comprising the step of harvesting said cells differentiated from said hematopoietic stem cells or progenitor cells from peripheral blood of a mammal prior to said transferring step, wherein said
5 transferring step occurs *in vitro* or *ex vivo*.

26. A method for treating a disorder in a mammal comprising the steps of delivering to said mammal a recombinant nucleic acid molecule comprising a first sequence comprising a transgene encoding a product under the
10 control of regulatory sequences that direct expression of the product of said transgene in a hematopoietic stem cell, a progenitor cell of the hematopoietic lineage, or a cell differentiated therefrom.

27. The method according to claim 26, wherein a differentiated cell
15 produces said product at a suitable site in said mammal.

28. The method according to claim 26, wherein delivering step comprises
(a) harvesting said stem cells or progenitor cells from bone marrow of said mammal; and
20 (b) transferring said nucleic acid molecule into said cells.

29. The method according to claim 28 further comprising reinfusing said cells into the bone marrow of said mammal.

25 30. The method according to claim 26, wherein delivering step comprises
(a) harvesting said differentiated cells from peripheral blood of said mammal; and
(b) transferring said nucleic acid molecule into said differentiated
cells.

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31. The method according to claim 30, further comprising reinfusing said cells into the blood of said mammal.

32. The method according to claim 26, wherein said delivering step
5 comprises administering said nucleic acid molecule directly into the mammal.

33. A method for treating or preventing thrombus formation in a mammal comprising delivering to a mammalian patient a recombinant nucleic acid molecule comprising a first sequence comprising a transgene encoding a fibrinolytic protein
10 under the control of regulatory sequences that direct expression of the product of said transgene in a platelet.

34. The method according to claim 33, wherein said fibrinolytic protein is selected from the group consisting of u-PA, Factor VIIa, Factor VIII, Factor IX and
15 fibrinogen.

35. The method according to claim 33, wherein said nucleic acid molecule is present in a platelet and said delivering step comprises administering to said patient said platelet.

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